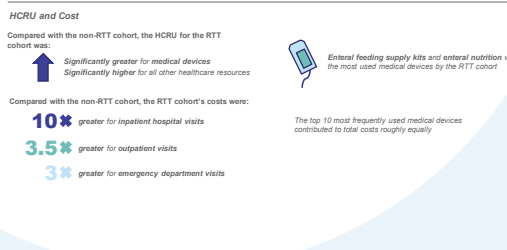
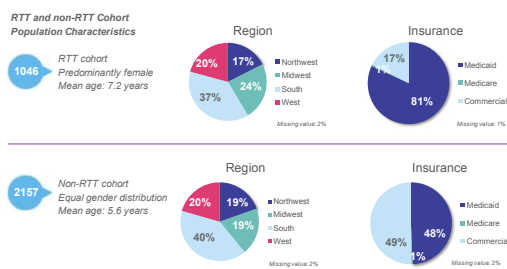
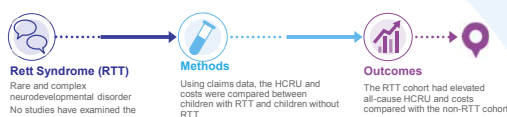


# Elevated Healthcare Resource Utilization and Costs Among Individuals Diagnosed With Rett Syndrome

Tigwa Davis<sup>1</sup>, Purva Parab<sup>1</sup>, Damian May<sup>2</sup>, Charles Ruetsch<sup>1</sup>

<sup>1</sup>Health Analytics, LLC; <sup>2</sup>Acadia Pharmaceuticals

## EXECUTIVE SUMMARY



## INTRODUCTION

- Rett syndrome (RTT) is a rare, complex, and progressive neurodevelopmental disorder<sup>1</sup>
- Management of the disorder often involves addressing symptoms through a multidisciplinary team-based approach<sup>2</sup>
- To date, this may be the first study to examine the healthcare resource utilization (HCRU) and costs of individuals diagnosed with RTT using a commercially available claims data set

## OBJECTIVES

- Compare the HCRU and medical costs between individuals with and without RTT

## METHODS

- Study Design**
- A retrospective cohort study using integrated medical claims from Clarivate's real-world data repository<sup>3</sup>
- Study Period**
- The study data represent years 2017 to 2022; the case-finding period was from June 1, 2018, to June 5, 2021
  - The baseline period was 12 months prior to the index date, and the follow-up period was 12 months after the index date
  - The index date was defined as the date of the first medical claim with a diagnosis of RTT (RTT cohort) or the date of the first medical claim (non-RTT cohort) during the case-finding period

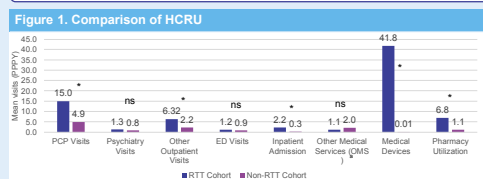
- Summary of Analytical Methods**
- Chi-square tests and t-tests were used to assess statistical differences for categorical and continuous variables, respectively, between the RTT and non-RTT cohorts. The alpha level for statistical significance was set at  $p < 0.05$

- Summary/List of Outcomes**
- Outcomes included HCRU and costs (per patient per year [PPPY])

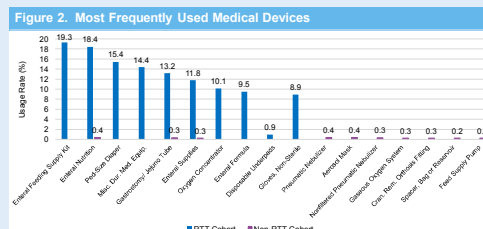
**Table 1. Comorbid conditions of the RTT and non-RTT cohorts**

	RTT cohort (n = 1046)		Non-RTT cohort (n = 2157)	
	n	%	n	%
Incontinence	435	41.6	29	1.3
Dysphagia	358	34.0	29	1.3
Speech/language disorders	358	34.0	183	8.5
Epilepsy/convulsions	338	32.3	35	1.6
Scoliosis	335	32.0	5	0.2
Convulsions	299	28.6	34	1.6
Constipation	296	28.3	123	5.7
Sleep disorders	289	27.7	70	3.2
GERD	191	18.3	54	2.5
Muscle weakness	142	13.6	13	0.6
Epilepsy	73	7.0	4	0.2
Dyspnea	69	6.6	44	2.0
Extrapyramidal movement	61	5.8	1	0.0
Anxiety	28	2.7	50	2.3
Long QT syndrome	26	2.5	0	0.0
Restlessness and agitation	20	1.9	9	0.4
Anorexia	19	1.8	51	2.4
Depression	2	0.2	5	0.2

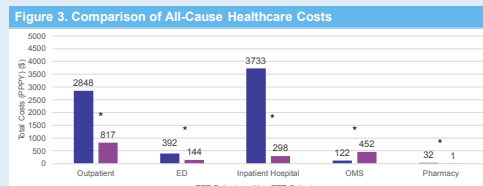
## RESULTS



\*Indicates statistically significant difference (p<0.05); ns, not significant. ED, emergency department; HCRU, healthcare resource utilization; CMS, other medical services; PCP, primary care physician; PPPY, per person per year; RTT, Rett syndrome



RTT, Rett syndrome



\*Indicates statistically significant difference (p<0.05). ED, emergency department; CMS, other medical services; PPPY, per person per year; RTT, Rett syndrome

**Figure 4. Costs for Medical Devices in the RTT Cohort**



Patients could be using more than one device at a time. PPPY, per person per year; RTT, Rett syndrome

## CONCLUSIONS

- Individuals with RTT required significantly more care from various physicians and allied health professionals
- The RTT cohort utilized more medical devices, resulting in significantly higher costs compared to the non-RTT cohort
- The RTT cohort had elevated all-cause service utilization and medical service utilization compared to the non-RTT cohort, which may result in considerable financial burden for payers, parents, and caregivers
- When individuals with RTT used healthcare resources, they had significantly higher costs compared to those who did not have RTT: service costs for the RTT cohort were 3 times (emergency department visits), 3.5 times (outpatient visits), and 10 times (inpatient hospital stays) greater than the non-RTT cohort

## LIMITATIONS

- The results are subject to the challenges routinely reported with the use of commercial claims data, such as the lack of information about disease severity, whether individuals met supportive criteria for a RTT diagnosis, or whether they were tested for a genetic mutation
- This study only used Clarivate's real-world data repository, as such, the results are not entirely generalizable due to using only one source of commercial claims data

## REFERENCES

- The National Institute of Neurological Disorders and Stroke. Rett Syndrome Fact Sheet. <https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Fact-Sheets/Rett-Syndrome-Fact-Sheet>. Accessed January 15, 2021.
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- Clarivate. Real World Data. <https://clarivate.com/products/real-world-data/>. Accessed January 15, 2021.

## ACKNOWLEDGMENTS

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## DISCLOSURES

Dr. May is an employee of Acadia Pharmaceuticals, Inc. which sponsored the study. Drs. Davis, Parab, and Ruetsch are employees of Health Analytics, LLC which was funded to conduct the study.

